

Rec'd PCT/PTO 15 APR 2005

10/532203

(12) INTERNATIONAL APPLICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

(19) World Intellectual Property
Organization
International Bureau



(43) International Publication Date
29 April 2004 (29.04.2004)

PCT

(10) International Publication Number
WO 2004/036181 A2

(51) International Patent Classification⁷: G01N

(74) Agents: LICATA, Jane, Massey et al.; Licata & Tyrrell
P.C., 66 East Main Street, Marlton, NJ 08053 (US).

(21) International Application Number:
PCT/US2003/033022

(81) Designated States (*national*): AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW.

(22) International Filing Date: 17 October 2003 (17.10.2003)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
60/419,392 17 October 2002 (17.10.2002) US

(84) Designated States (*regional*): ARIPO patent (GH, GM, KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian patent (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European patent (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PT, RO, SE, SI, SK, TR), OAPI patent (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).

(71) Applicant (*for all designated States except US*): THE TRUSTEES OF THE UNIVERSITY OF PENNSYLVANIA [US/US]; Center for Technology Transfer, Suite 200, 3160 Chestnut Street, Philadelphia, PA 19104-6283 (US).

Published:

— without international search report and to be republished upon receipt of that report

(72) Inventors; and

(75) Inventors/Applicants (*for US only*): DREYFUSS, Gideon [US/US]; 343 Aubrey Road, Wynnewood, PA 19096 (US). WANG, Jin [CN/US]; 608 Sturwood Way, Lawrenceville, NJ 08648 (US).

For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.



WO 2004/036181 A2

(54) Title: METHOD OF TREATING NEUROLOGICAL DISEASES AND DISORDERS

(57) Abstract: The present invention provides methods for treating neurological diseases and disorders. Compounds that replace or enhance the function of SMN or alleviate or reduce a phenotype of cells with low SMN protein levels are provided. Screening methods and kits for identifying such compounds also are provided.